FAMILIESUSA
THE VOICE FOR HEALTH CARE CONSUMERS

March 04, 2016

The Hon. Charles Grassley
The Hon. Ron Wyden
Committee on Finance
United States Senate
Washington, DC 20510

Dear Senator Grassley and Senator Wyden,

Families USA appreciates the opportunity to offer comments on the issues and questions raised in the report "The Price of Sovaldi and Its Impact on the U.S. Health Care System." Families USA is a national, non-partisan, non-profit consumer advocacy organization dedicated to the achievement of high quality, affordable care for all in this country.

We commend your leadership and the work of your staff in conducting this 18-month investigation into the pricing of the new, breakthrough treatments for hepatitis C. We are very concerned about the impacts of these high-cost drugs, as well as the overall cost of prescription drugs, on consumers, their families, and the health care system as a whole.

We look forward to continued work with the Committee and others to address this issue. If you have any questions about our comments, please contact Ellen Albritton, Policy Analyst, at ealbritton@familieusa.org, or Caitlin Morris, Program Director for Health System Transformation, at cmorris@familiesusa.org.

Sincerely,

Ellen Albritton
Policy Analyst
Families USA

FamiliesUSA.org
1201 New York Avenue, NW, Suite 1100
Washington, DC 20005
main 202-628-3030 / fax 202-347-2417

What are the effects of a breakthrough, single source innovator drug on the marketplace?

As seen with the releases of Sovaldi and Harvoni, breakthrough single-source innovator drugs can put immense pressure on federal and state health care program budgets, lead private and public payers to enact burdensome access restrictions, and/or impose significant financial barriers for patients who could otherwise benefit from the drug. As an increasing percentage of drugs under development are high-cost biologics, it is vitally important that competitor drugs are brought to the market as soon as possible, while still ensuring the safety and efficacy of the drugs. For example, the Committee may explore ways to expedite the approval pathway for biologics with only one or two competitors on the market, or to reduce the market exclusivity period for biologics.

As important as competition can be for lowering the price of drugs, we are concerned that drug manufacturers may set a higher launch price in order to set up even higher prices for subsequent drugs and that competing drugs may still be priced just slightly below existing drugs on the market. We believe that greater transparency regarding research and development (R&D) costs, clinical trial results, and final negotiated prices, and the consideration of a drug's relative value to patients in its pricing, as described in more detail below, can help ensure that innovative drugs remain accessible to those who need and can benefit from them.

Do payers in the programs have adequate information to know the cost, patient volume, and increases in efficacy of a new treatment regimen?

Both private and public payers often do not have adequate information to understand the cost of new treatments, who and how many patients are eligible for the treatment, and the increased clinical benefits the treatment may bring to patients as compared to existing alternatives. This information is valuable not only to payers, but also to patients and providers. Sustained and robust federal investments in research, from basic research through health services and comparative effectiveness research (CER), are necessary to ensure that this vital information is known and made available. Equally important is increased enforcement of existing requirements for clinical trial reporting. Studies have shown that only between 13% and 22% of trials subjected to mandatory reporting under the Food and Drug Administration Amendments Act had met requirements for reporting trial results to ClinicalTrials.gov. With so much missing data, our understanding of a drug's safety, efficacy, and impact on different patient populations is incomplete.

Although CER is already being conducted by drug manufacturers, insurers, and others, in order for this research to be the most effective, we recommend that this research be organized, aggregated, and validated by a single, independent entity that would serve as a CER "clearinghouse." Research conducted by other entities, including that done by manufacturers for submission to foreign regulators, would be required to be submitted to this organization, which would also conduct its own research and analyses. For each newly approved drug, this independent entity would evaluate its added benefits over existing drugs and therapies and would

FamiliesUSA.org
1201 New York Avenue, NW, Suite 1100
Washington, DC 20005
main 202-628-3030 / fax 202-347-2417

release its findings in a standardized, consumer-friendly manner, such as a star-rating system, that could easily be understood by payers, providers, and patients.

What roles does the concept of "value" play in this debate, and how should an innovative therapy's value be represented in its price?

With spending on prescription drugs rising faster than any other health care item or service, it is important that the prices we are paying truly reflect the value of new drugs and the added benefits they bring to the patients who need them. The comparative effectiveness ratings produced by an independent entity (described above) can be used to help align drug prices with their actual added value. This organization can produce recommended price ranges based on a new drug's added benefit. This additional transparency regarding the added benefits and recommend pricing can be of great value for payers in negotiating the final price with drug manufacturers. Federal regulators can consider additional incentives for drug manufacturers to stay within the recommended price ranges.

We also recommend that the Committee consider different "pay for value" mechanisms that can ensure drugs are as effective in improving real-world outcomes as they promise to be following clinical trials. One such model, known as "pay for success," includes an initial payment for a drug based on the cost of existing drugs, with supplemental payments if the drug is successful in improving outcomes over the previous drugs. We recommend the Center for Medicare & Medic aid Innovation test how this model could be implemented and for which drugs it would be most appropriate.

The Food and Drug Administration (FDA) can also take steps to encourage more value-based payments across both public and private payers. Often, drugs are used to treat several different conditions, as is the case with many high-cost cancer drugs being used to treat different types of cancers. Payers pay the same price regardless of what condition a drug is being used to treat, even though the drugs may differ considerably in their effectiveness across the different conditions. While some private payers are beginning to experiment with indication-specific pricing, it remains challenging to do so, as National Drug Codes (NDCs) assigned by the FDA do not specify use or indication. By issuing NDCs that differentiate for different uses of a drug, the FDA could remove a major barrier to more payers utilizing this value-based payment.

Additionally, we encourage the Department of Health and Human Services (HHS) to issue guidance on federal agencies' "march-in" rights to patents derived from federally-funded research, which were authorized under the Bayh-Dole Act. The National Institutes of Health and other federal agencies use taxpayer dollars to make substantial investments in research, from which drug manufacturers benefit considerably. We believe that such guidance from HHS can help guard against drug manufacturers setting unreasonably high prices that result in drugs not being "available to the public on reasonable terms" or prevent health and safety needs from being met, as specified in the Bayh-Dole Act.

What measures might improve price transparency for new higher-cost therapies while maintaining incentives for manufacturers to invest in new drug development?

The complex nature of payment for prescription drugs makes it challenging, if not impossible, for payers, providers, and consumers to ever know or adequately predict the prices they will pay for prescription drugs. As a major purchaser of these drugs, the federal government has a strong interest in knowing how drug prices, especially those for higher-cost therapies, will impact various federal health care programs. We encourage the Committee to explore mechanisms for requiring drug manufacturers to disclose their final negotiated prices, inclusive of rebates, with different payers, pharmacy benefit managers, and federal and state health care programs, as well as the prices negotiated with other countries. Having more complete pricing information will help policymakers and the public to assess how the costs of these drugs are distributed across the health care system.

In addition to increased price transparency, we also encourage the Committee to explore mechanisms to require drug manufacturers to disclose information related to their R&D investments and marketing and advertising expenditures. This information is necessary for ensuring that the prices of drugs reflect the true costs of R&D and that profits earned from these drugs are reinvested in new R&D. We recommend that pharmaceutical companies be required to disclose the following: total cost of production of a drug; R&D costs for specific drugs, including details on R&D funded via public funds; marketing spending for a drug; and total profit made from a drug.

What tools exist, or should exist, to address the impact of high cost drugs and corresponding access restrictions, particularly on low-income populations and state Medicaid programs?

Though we are concerned about the budgetary pressures put on state Medicaid programs as a result of these high-cost drugs, we do not support states enacting access restrictions that would prevent or seriously delay patients' access to drugs that can bring them enormous clinical benefit and improved quality of life. We commend the Centers for Medicare & Medicaid Services (CMS) for issuing guidance instructing states to stop limiting access to the new hepatitis C drugs, and we encourage CMS to continue to monitor access and take additional enforcement action, if necessary, to ensure state Medicaid programs are in compliance with federal laws and regulations. As Medicaid beneficiaries have low incomes, very few have the resources to pay for these drugs, or any other high-cost drugs, out-of-pocket. The same is true for low-income individuals with marketplace or employee-sponsored health insurance, and we are very concerned that such access restrictions could lead to increased health disparities for vulnerable populations. We encourage the Committee to pursue mechanisms that protect Medicaid beneficiaries and other vulnerable consumers from access restrictions or cost-sharing that would prevent them from receiving these potentially life-saving drugs.

To address the impact of high cost drugs on both public and private payers and consumers, we strongly encourage the Committee to pursue solutions that increase transparency regarding the prices and added benefits of new drugs, that incentivize drug manufacturers to align drug prices with relative benefit, and that encourage broader "pay for value" models.